

## TITLE

### **Interim Analysis of Safety, Pharmacology, and Immunogenicity of 4 weekly doses of PRTX-100 in adult patients with ITP**

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## ABSTRACT

A phase Ib, ascending dose safety and pharmacology study of PRTX-100 (purified staphylococcal protein A) is being performed at seven hematology sites in patients with chronic immune-mediated thrombocytopenia (ITP). The study will dose sequential cohorts of patients with 0.075, 0.15, or 0.30 µg/kg of PRTX-100, given intravenously once weekly for 4 weeks, and is currently enrolling the second cohort (dose of 0.15 µg/kg per week). All treatment-emergent adverse events to date were of mild or moderate severity. To date, with 9 patients dosed, 10 AE's which were considered possibly CTM-related have been noted. Eight AE's were mild. The two possibly-related AE's of moderate severity were abdominal pain related to bloating and gas in one patient and shoulder and back myalgias in a second patient. Of interest, one patient had had "rigors and chills" rated of mild severity during the evening after the first dosing, and considered possibly-related to CTM. This patient also had a significant elevation of CRP after the first dosing, to a peak of 38.7 mg/L after 48 hrs, returning to normal (5.11 mg/L) by study day 7. This laboratory finding was recorded as an AE of mild severity. This patient also reported pain of the R axilla 48 hrs after dosing, rated mild in severity, considered possibly related. This patient was pretreated with paracetamol prior to subsequent dosing and did not experience further peri-dosing AE's or further CRP elevations with the next three doses. In general, peri-dosing AE's have been uncommon and mild or moderate with repeated PRTX-100 dosing, and CTM-related laboratory abnormalities have been limited to a transient elevation of the C-reactive protein in one patient. Pharmacokinetic data thus far suggest that C<sub>max</sub> and AUC are consistent with predictions from single-dose studies. To date approximately half of patients have developed anti-PRTX-100 antibodies by study day 28.